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Public summary of opinion on orphan designation

Temozolomide for the treatment of neuroblastoma

On 21 August 2019, orphan designation EU/3/19/2188 was granted by the European Commission to ORPHELIA Pharma S.A.S, France, for temozolomide for the treatment of neuroblastoma.

What is neuroblastoma?

Neuroblastoma is a cancer of certain nerve cells which is usually seen as a lump in the abdomen or around the spine. Symptoms may include weakness, bone pain, loss of appetite and fever.

Neuroblastoma is the most common solid tumour outside the brain in children. In many cases it is present at birth but is diagnosed later when the cancer has spread to other parts of the body and the child begins to show symptoms of the disease.

Neuroblastoma is a long-term debilitating and life-threatening disease that is associated with poor long-term survival.

What is the estimated number of patients affected by the condition?

At the time of designation, neuroblastoma affected approximately 1.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 57,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, several methods were used in the EU for treating neuroblastoma, including surgery, chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation). The medicine Qarziba (dinutuximab beta) was authorised for patients with high-risk neuroblastoma (which has a high chance of coming back).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with neuroblastoma because early studies found that the condition improved in

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 518,400,000 (Eurostat 2019).

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patients for whom other treatments did not work or had stopped working. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Temozolomide is a cancer medicine that has been authorised in the EU for several years to treat glioma. It belongs to a group of cancer medicines called alkylating agents. In the body, temozolomide is converted into another substance called methyl diazonium ion, which damages the DNA of cells. This stops tumour cells in the brain from reproducing and slows down the growth of the neuroblastoma.

What is the stage of development of this medicine?

The effects of temozolomide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with temozolomide in patients with neuroblastoma were ongoing.

At the time of submission, temozolomide was not authorised anywhere in the EU for the treatment of neuroblastoma or designated as an orphan medicinal product elsewhere for this condition. Temozolomide was authorised in the EU for the treatment of glioma under the name Temodal.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 July 2019, recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Temozolomide	Treatment of neuroblastoma
Bulgarian	Темозоломид	Лечение на невробластом
Croatian	Temozolomid	Liječenje neuroblastoma
Czech	Temozolomid	Léčba neuroblastomu
Danish	Temozolomid	Behandling af neuroblastom
Dutch	Temozolomide	Behandeling van neuroblastoom
Estonian	Temosolomiid	Neuroblastoomi ravi
Finnish	Temotsolomidi	Neuroblastooman hoito
French	Témozolomide	Traitement du neuroblastome
German	Temozolomid	Behandlung des Neuroblastoms
Greek	Τεμοζολομίδη	Θεραπεία του νευροβλαστώματος
Hungarian	Temozolomid	Neuroblastoma kezelése
Italian	Temozolomide	Trattamento del neuroblastoma
Latvian	Temozolomīds	Neiroblastomas ārstēšana
Lithuanian	Temozolomidas	Neuroblastomos gydymas
Maltese	Temozolomide	Kura tan-newroblastoma
Polish	Temozolomid	Leczenie nerwiaka płodowego
Portuguese	Temozolomida	Tratamento do neuroblastoma
Romanian	Temozolomidă	Tratamentul neuroblastomului
Slovak	Temozolomid	Liečba neuroblastómu
Slovenian	Temozolomid	Zdravljenje nevroblastoma
Spanish	Temozolomida	Tratamiento del neuroblastoma
Swedish	Temozolomid	Behandling av neuroblastom
Norwegian	Temozolomid	Behandling av nevroblastom
Icelandic	Temózólómíð	Meðferð við taugakímfrumuæxli

[1] At the time of designation